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=> d his

     (FILE 'HOME' ENTERED AT 15:58:38 ON 11 MAY 2009)

     FILE 'MEDLINE, CAPLUS, BIOSIS, SCISEARCH, LIFESCI' ENTERED AT 15:59:09 ON
     11 MAY 2009
L1          240 S (REDUC? OR DECREAS? OR INHIBIT?)(5A)(GL-3 OR GLOBOTRIAOSYLCER
L2            3 S (AAV OR ADENO-ASSOCIATED(W)VIRUS)(7A)ALPHA-GALACTOSIDASE(W)A
L3            7 S (AAV OR ADENO-ASSOCIATED(W)VIRUS)(7A)ALPHA-GALACTOSIDASE
L4            2 S L1 AND L3
L5            2 DUP REM L4 (0 DUPLICATES REMOVED)

=> d au ti so pi 1-2 l5

L5   ANSWER 1 OF 2  CAPLUS  COPYRIGHT 2009 ACS on STN
IN   Cheng, Seng H.; Meeker, David
TI   Combined enzyme replacement, gene therapy and small molecule therapy for
     lysosomal storage diseases
SO   U.S. Pat. Appl. Publ., 35 pp., Cont.-in-part of U.S. Ser. No. 884,526.
     CODEN: USXXCO
     PATENT NO.          KIND   DATE        APPLICATION NO.          DATE
     ---------------     ----   --------    -------------------      --------
PI   US 20040204379      A1     20041014    US 2004-758773           20040116
     US 20020095135      A1     20020718    US 2001-884526           20010619
     US 20070280925      A1     20071206    US 2007-762689           20070613

L5   ANSWER 2 OF 2  CAPLUS  COPYRIGHT 2009 ACS on STN
AU   Takahashi, Hiroshi; Hirai, Yukihiko; Migita, Makoto; Seino, Yoshihiko;
     Fukuda, Yuh; Sakuraba, Hitoshi; Kase, Ryoichi; Kobayashi, Toshihide;
     Hashimoto, Yasuhiro; Shimada, Takashi
TI   Long-term systemic therapy of Fabry disease in a knockout mouse by
     adeno-associated virus-mediated muscle-directed gene transfer
SO   Proceedings of the National Academy of Sciences of the United States of
     America (2002), 99(21), 13777-13782
     CODEN: PNASA6; ISSN: 0027-8424


=> d ab 2 l5

L5   ANSWER 2 OF 2  CAPLUS  COPYRIGHT 2009 ACS on STN
AB   Fabry disease is a systemic disease caused by genetic deficiency of a
     lysosomal enzyme, α-galactosidase A (α-gal A), and is thought
     to be an important target for enzyme replacement therapy.  We studied the
     feasibility of gene-mediated enzyme replacement for Fabry disease.  The
     adeno-associated virus (AAV) vector containing the α-gal A gene was
     injected into the right quadriceps muscles of Fabry knockout mice.  A time
     course study showed that α-gal A activity in plasma was increased to
     ≈25% of normal mice and that this elevated activity persisted for
     up to at least 30 wk without development of anti-α-gal A antibodies.
     The α-gal A activity in various organs of treated Fabry mice
     remained 5-20% of those observed in normal mice.  Accumulated
     globotriaosylceramide in these organs was completely cleared by 25 wk
     after vector injection.  Redn. of globotriaosylceramide
     levels was also confirmed by immunohistochem. and electronmicroscopic
     analyses.  Echocardiog. examination of treated mice demonstrated structural
     improvement of cardiac hypertrophy 25 wk after the treatment.  AAV
     vector-mediated muscle-directed gene transfer provides an efficient and
     practical therapeutic approach for Fabry disease.


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